EXHIBIT E

American Society of Gene & Cell Therapy

Final Program Addendum

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Late Abstracts

800. The First Report on a rAAV.sFlt-1 Phase I/II Trial for Wet Age-Related Macular Degeneration (AMD) EP Rakoczy¹, CM Lai¹, C Pierce², A Magno², D Dismuke³, J Grieger³, RJ Samulski³, TW Chalberg⁴, SD Schwartz⁵, MS Blumenkranz⁶, IJ Constable¹

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The standard of care in treating wet AMD involves frequent intraocular injection of recombinant anti-VEGF proteins every 4-8 weeks. We developed a rAAV construct for a potent (Kd ~10 pM), naturally occurring anti-VEGF protein, soluble Fms-related tyrosine kinase-1 (sFlt-1), for the treatment of wet AMD. rAAV.sFlt-1 was produced in accordance with FDA and ICH guidelines at the UNC Vector Core Human Application Laboratory. Here we report on three patients who received rAAV.sFlt-1 in January 2012. The three enrolled subjects (mean age 77 years) all had active subfoveal choroidal neovascularization, with visual acuity of 20/80 to 20/400, and had previously received multiple intravitreal injections of ranibizumab. A final injection of ranibizumab was given at baseline, and on day 7, 1 x 10E10 vector genomes of rAAV.sFlt-1 in 100 ul volume was administered via subretinal injection. In all three cases, the bleb of sub-retinal fluid resolved within 4 hours. After 24 hours, most of the air in the vitreous had absorbed and only the retinal injection site remained visible. One patient developed a minor hemorrhage associated with the procedure that did not affect vision. As expected following vitrectomy, there was a transient increase in neutrophil counts that returned to normal by 14 days post injection. Vector sequence was found in the tears of one subject at one day post injection that cleared by day 30. Other than this single occurrence, AAV2 was not detected in any of the subjects' blood, saliva or urine samples either by qPCR or ELISA to date. Background levels of the naturally occurring sFlt-1 protein showed a high baseline variation in the urine, serum, and saliva with no increase following treatment. sFlt-1 levels in the vitreous also varied among subjects (975-2085pg/ml). Blood biochemistry, complete blood count, and T-cell response, remained without any significant change compared to baseline. Subretinal injection of rAAV.sFlt-1 showed no clinically significant retinal toxicity as assessed by serial ophthalmic examinations over a two month period. No superficial, anterior segment or vitreous inflammatory signs were present in any of the subjects. At day 60 none of the patients required rescue treatment. There was no evidence of visual acuity loss, IOP elevation, retinal detachment, or any intraocular or systemic immune response in any of the patients. These results extend the promise of gene therapy for the large cohort of elderly patients (>75) suffering from wet AMD. Further efficacy results with the low dose (1 x 10E10 vg) cohort and safety on the high-dose (1 x 10E11 vg) cohort will be discussed.

801. Long-term airway epithelial transduction and induction of tolerance to transgene via intra-tracheal administration of airway-targeting lentivirus to mid-gestation fetal sheep

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Prenatal gene transfer to the respiratory epithelium may provide opportunities to overcome physical and immunological barriers limiting efficient gene transfer to the adult lung. Unlike the adult, airway stem and progenitor cells are; (1) present at higher frequencies during fetal life; and (2) more accessible to gene transfer as the airways are devoid of mucous and pulmonary macrophages. In addition, the immature immune system of the fetus provides the possibility of inducing immunological tolerance to the exogenous vector and transgene that might allow repeated postnatal delivery of the same vector / transgene. We recently demonstrated that a lentivirus (HIV-1-based) pseudotyped with Jaagsiekte sheep retrovirus envelop (JSRV-LV) expressing GFP administered into the fetal lung lumen at ~70days of gestation (full term ~145days) transduced ~30% of the fetal airways, between 100-300µm diameter, when examined 10 weeks post-transduction ⁽¹⁾. We examined the durability of the respiratory cell transduction 6 months after birth and analyzed immunological tolerance to GFP

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